PhaseBio Provides Pemziviptadil (PB1046) Program Update

October 23, 2020

VANGARD trial discontinued due to evolving COVID-19 treatment landscape, recent feedback from FDA regarding regulatory and development path, and interim analysis of trial data

Enrollment expected to resume for ongoing Phase 2b trial of pemziviptadil in pulmonary arterial hypertension (PAH)

MALVERN, Pa. & SAN DIEGO--(BUSINESS WIRE)--Oct. 23, 2020-- PhaseBio Pharmaceuticals, Inc. (Nasdaq: PHAS), a clinical-stage biopharmaceutical company focused on the development and commercialization of novel therapies for cardiopulmonary diseases, today announced that, after a strategic review of the VANGARD clinical trial that included an assessment of the evolving COVID-19 treatment landscape, feedback from the U.S. Food and Drug Administration (FDA) and an interim analysis of the VANGARD study data, it has elected to discontinue the trial. Importantly, pemziviptadil was generally well tolerated and there was no adverse safety signal reported in the VANGARD trial.

In response to the medical community's rapidly evolving understanding of COVID-19 disease progression and approaches to treatment, PhaseBio recently submitted a revised VANGARD trial protocol and received feedback from the FDA regarding the regulatory and development path in COVID-related ARDS. For pemziviptadil use in hospitalized COVID-19 patients at high risk for rapid clinical deterioration and ARDS, the FDA highlighted the likely need for additional clinical trials with sufficient sample size to adequately assess mortality risk. Based on this feedback, PhaseBio determined that it would be unlikely that the 70 patients targeted for enrollment in each of the VANGARD trial's treatment arms would be sufficient in size to adequately evaluate mortality and that at least one additional clinical trial with a mortality endpoint, which would require significantly more patients, would be required for approval of pemziviptadil for use in patients diagnosed with COVID-19.

PhaseBio also elected to accelerate the timeline for its previously planned interim analysis. Based upon its analysis of the first 25 patients enrolled in the VANGARD trial, PhaseBio did not observe any data trends in the 40 mg or 100 mg pemziviptadil-treated arms of the trial that suggested a reasonable probability of achieving the primary efficacy endpoint; however, data from a larger number of patients would be necessary to draw a definitive conclusion. Pemziviptadil was generally well tolerated, and an independent data safety monitoring board did not identify any safety concerns related to pemziviptadil.

"While extremely difficult to make, our decision to discontinue the VANGARD trial came after a review of the rapidly evolving COVID-19 treatment landscape, the changing regulatory path, the results of the interim data analysis and the potential benefit to patients of advancing this program versus deploying our resources to other studies," said Jonathan Mow, Chief Executive Officer, PhaseBio Pharmaceuticals. "We would like to thank the patients who participated in the VANGARD trial, the trial investigators and the PhaseBio team, who worked tirelessly to rapidly design and launch the trial during a global pandemic. We remain committed to moving our broader pipeline forward, fulfilling our strategic objectives and delivering new treatment options for patients with serious cardiovascular diseases."

The ongoing Phase 2b trial of pemziviptadil in patients with PAH, named the VIP Trial (**V**asoactive **I**ntestinal **P**eptide in adult patients with pulmonary arterial hypertension) is expected to quickly resume enrollment after a pause related to the impacts of the COVID-19 pandemic and re-prioritization of drug supply to the VANGARD trial. To date, approximately one third of the patients targeted for enrollment have completed the initial 16 week protocol, with approximately 90% of these patients electing to enroll in VIP EXTEND (Vasoactive Intestinal Peptide extension trial in adult patients with pulmonary arterial hypertension), the open label extension of the Phase 2b trial. Results from the VIP Trial are expected to be reported in the second half of 2021.

About Pemziviptadil (PB1046)

Pemziviptadil, a novel, subcutaneously-injected vasoactive intestinal peptide (VIP) analogue, is a recombinant fusion protein composed of VIP and PhaseBio's proprietary elastin-like polypeptide (ELP) biopolymer. Based on the pharmacokinetic profile of pemziviptadil observed in clinical trials, the fusion of VIP to ELP results in both a prolonged absorption profile and a longer circulating half-life, enabling once-weekly dosing.

Pemziviptadil is in Phase 2 development for the treatment of pulmonary arterial hypertension (PAH). PhaseBio expects to report initial data from the Phase 2b trial in PAH in the second half of 2021. To date, pemziviptadil has been administered to more than 100 patients with cardiovascular or cardiopulmonary diseases in five clinical trials conducted in the United States. The FDA has granted pemziviptadil orphan drug designation for the treatment of pulmonary arterial hypertension (WHO Group 1 Pulmonary Hypertension) and cardiomyopathy associated with dystrophinopathies.

About PhaseBio Pharmaceuticals

PhaseBio Pharmaceuticals, Inc. is a clinical-stage biopharmaceutical company focused on the development and commercialization of novel therapies for cardiovascular and cardiopulmonary diseases. The company's pipeline includes: bentracimab (PB2452), a novel reversal agent for the antiplatelet therapy ticagrelor; pemziviptadil (PB1046), a once-weekly vasoactive intestinal peptide receptor agonist for the treatment of pulmonary arterial hypertension; and PB6440, an oral agent for the treatment of resistant hypertension. PhaseBio's proprietary elastin-like polypeptide technology platform enables the development of therapies with potential for less-frequent dosing and improved pharmacokinetics, including pemziviptadil, and drives both internal and partnership drug-development opportunities.

PhaseBio is located in Malvern, PA, and San Diego, CA. For more information, please visit www.phasebio.com.

Forward-Looking Statements

This press release contains forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995. Words such as "anticipates," "believes," "expects," "intends," "projects," and "future" or similar expressions are intended to identify forward-looking statements. Forward-looking statements include statements concerning or implying the conduct or timing of our Phase 2b trial of pemziviptadil for the treatment of pulmonary arterial hypertension, including the expected timing for resuming enrollment and reporting results from such trial, or other product candidates in our pipeline, our research, development and regulatory plans for our product candidates, the potential for these product candidates to receive regulatory approval from the FDA or equivalent foreign regulatory agencies, and

whether, if approved, these product candidates will be successfully distributed and marketed. Forward-looking statements are based on management's current expectations and are subject to various risks and uncertainties that could cause actual results to differ materially and adversely from those expressed or implied by such forward-looking statements. Accordingly, these forward-looking statements do not constitute guarantees of future performance, and you are cautioned not to place undue reliance on these forward-looking statements. Risks regarding our business are described in detail in our Securities and Exchange Commission filings, including in our Quarterly Report on Form 10-Q for the quarter ended June 30, 2020. Such risks may be amplified by the impacts of the COVID-19 pandemic. These forward-looking statements speak only as of the date hereof, and PhaseBio Pharmaceuticals, Inc. disclaims any obligation to update these statements except as may be required by law.

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